

दुर्मिळ आजारावरील औषधोपचारासाठी
राष्ट्रीय धोरण- २०२१ व राष्ट्रीय आरोग्य
निधीची छत्र योजना (Umbrella Scheme
of Rashtriya Arogya Nidhi- RAN)
याच्या अंमलबजावणीबाबत मार्गदर्शक
सूचना.

महाराष्ट्र शासन
सार्वजनिक आरोग्य विभाग
शासन परिपत्रक क्रमांक: दुर्मिळ आजार-२०२०/प्र.क्र.२१/आरोग्य-७
१० वा मजला, गो. ते. रुग्णालय संकूल इमारत,
नवीन मंत्रालय, मुंबई - ४००००१.
दिनांक : १७ ऑगस्ट, २०२१.

वाचा :-

१. केंद्र शासन, आरोग्य व कुटुंब कल्याण मंत्रालयाचे कार्यालयीन ज्ञापन क्र.F.No.११०३६/३९/
२०१७-RAN, दिनांक ०१.०२.२०१९ व दिनांक ३०.१०.२०१९.
२. केंद्र शासन, आरोग्य व कुटुंब कल्याण मंत्रालयाचे कार्यालयीन ज्ञापन क्र.F.No.१२०१४/१०८/
२०२०-Grants, दिनांक ३०.०३.२०२१.

परिपत्रक:-

उपरोक्त दिनांक ०१.०२.२०१९ च्या कार्यालयीन ज्ञापनाद्वारे राष्ट्रीय आरोग्य निधीच्या नवीन
छत्र योजनेखाली दारिद्र्य रेषेखालील व्यक्तींना (Persons below threshold poverty line)
विनिर्दिष्ट दुर्मिळ आजारावरील उपचारासाठी एका वेळेच्या वित्तीय सहाय्याची योजना विहित करण्यात
आली आहे. तसेच केंद्र शासनाच्या दिनांक ३०.१०.२०१९ च्या कार्यालयीन ज्ञापनान्वये उपरोक्त
मार्गदर्शक सूचनांमध्ये काही सुधारणा करण्यात आल्या आहेत. सदर दोन्ही कार्यालयीन ज्ञापन सुलभ
संदर्भासाठी सोबत जोडले आहेत. (सहपत्र १ व २)

०२. सुधारित योजनेत ठराविक शासकीय रुग्णालयात यासाठी केंद्र शासनाने आवर्ती परिक्रमण निधी
(Revolving Fund) स्थापन केला असून पात्र रुग्णांच्या उपचारांच्या रुपये पाच लाख पर्यंतच्या
उपचारासाठी आवर्ती परिक्रमण निधीमधून खर्च करण्याचे अधिकार असा निधी स्थापन करण्यात
आलेल्या रुग्णालयांच्या वैद्यकीय अधीक्षक/ संचालक यांना प्रत्यावर्तीत करण्यात आले आहेत.

आवर्ती परिक्रमण निधी स्थापन केलेल्या रुग्णालयातील रुपये पाच लाख पेक्षा जास्त रकमेची
प्रकरणे व परिक्रमण निधी स्थापन न केलेल्या रुग्णालयांची सर्व वित्तीय सहाय्याची प्रकरणे मान्यतेसाठी

केंद्रीय आरोग्य व कुटुंब कल्याण मंत्रालयाला संदर्भित करावयाची आहेत. अशा रुग्णालयांना मंत्रालयामार्फत मंजूरी आदेशासह निधी वितरण करण्यात येईल.

सदर योजनेचा लाभ दारिद्र्य रेषेखालील जे रुग्ण अति विशेष उपचार सेवा असलेल्या (Super specialty facilities) शासकीय रुग्णालयात उपचार घेत आहेत अशानाच अनुज्ञेय आहे. खासगी रुग्णालयात उपचार घेत असलेल्या रुग्णांना या योजनेचा लाभ अनुज्ञेय नाही.

०३. आरोग्य व कुटुंब कल्याण मंत्रालयाने जुलै, २०१७ मध्ये दुर्मिळ आजारांवरील उपचारांचे धोरण- २०१७ विहित केले असून तथापि सदर धोरणाच्या अंमलबजावणीमध्ये येणाऱ्या अडचणी विचारात घेऊन सदर धोरण स्थगित करण्यात आले होते. तद्नंतर या संदर्भात प्राप्त झालेल्या सूचना व शिफारशी विचारात घेऊन सन २०१७ मधील धोरणाचा फेरआढावा घेण्यात आला असून आता दिनांक ३०.०३.२०२१ च्या कार्यालयीन जापनाद्वारे दुर्मिळ आजारांवरील उपचारांचे राष्ट्रीय धोरण २०२१ हे आरोग्य व कुटुंब कल्याण मंत्रालयाने निश्चित करून प्रसिध्द केले आहे. सदर धोरणाची प्रत सुलभ संदर्भासाठी सोबत जोडली आहे. (सहपत्र ३)

०४. नवीन धोरणाची प्रमुख वैशिष्ट्ये पुढीलप्रमाणे आहेत.:-

➤ दुर्मिळ आजारांचे तीन गटात वर्गीकरण:-

Group 1: Disorders amenable to one-time curative treatment

Group 2: Diseases requiring long term/ lifelong treatment having relatively lower cost of treatment and benefit has been documented in literature and annual or more frequent surveillance is required.

Group 3: Diseases for which definitive treatment is available but challenges are to make optimal patient selection for benefit, very high cost and lifelong therapy.

➤ विविध स्तरावर प्रतिबंधात्मक उपाययोजना

➤ प्रकर्ष केंद्र (Centres of Excellence) व निदान केंद्र यांची स्थापना :-

दुर्मिळ आजारांचे निदान व उपचार यासाठी शासकीय दर्शरी रुग्णालय म्हणून केंद्र शासन प्रकर्ष केंद्रे (Centers of Excellence) अधिसूचित करणार आहे. यामध्ये राज्यातील के.ई.एम.रुग्णालय, मुंबई याचा समावेश आहे.

याशिवाय जैव तंत्रज्ञान विभागाकडून Unique Methods of Management and Treatment of Inherited Disorders (UMMID) या प्रकल्पांतर्गत अनुवांशिक तपासणी व सल्लागार सेवा (Genetic Testing and Counseling) यासाठी निदान केंद्रे स्थापन करण्यात येणार आहेत. सदर निदान केंद्रे प्रकर्ष केंद्रांच्या मार्गदर्शन व पर्यवेक्षणाखाली कार्यरत राहतील. सद्यस्थितीत राज्यात निदान केंद्र देण्यात आलेले नाही. तथापि, CMC Vellore ही सदुपदेशक संस्था (Mentor Institute) राज्यातील वाशिम या “आकांक्षित” जिल्ह्यात दुर्मिळ आजारांसाठी सहाय्यभूत संस्था म्हणून केंद्र शासनाने निश्चित केली आहे.

➤ **दुर्मिळ आजारावरील उपचारासाठी शासकीय सहाय्य:-**

गट-१ मधील दुर्मिळ आजार ज्यांवर एकदाच उपचार करणे आवश्यक आहे अशासाठी रु.२०.०० लाखापर्यंतचे वित्तीय सहाय्य राष्ट्रीय आरोग्य निधीमधून केंद्र शासनामार्फत उपलब्ध करून दिले जाईल. या वित्तीय सहाय्यासाठी फक्त दारिद्र्य रेषेखालील लाभार्थीच पात्र नसून, प्रधानमंत्री जन आरोग्य योजनेच्या निकषाप्रमाणे पात्र ठरणार्या व केवळ शासकीय दर्शरी रुग्णालयात उपचारासाठी ४० टक्के लोकसंख्येला हे लाभ दिले जातील.

गट-२ मधील दुर्मिळ आजारांच्या रुग्णांना सहाय्य देण्याबाबत राज्य शासनाने विचार करावा, असे सदर धोरणात सूचविण्यात आले आहे.

गट-३ मधील दुर्मिळ आजारांच्या उपचारांवर येणारा प्रचंड खर्च व संसाधनांची मर्यादा आणि सार्वजनिक आरोग्याची इतर प्राधान्ये विचारात घेऊन अशा आजारांवर रुग्ण ज्या रुग्णालयात उपचार घेत आहेत आणि अशा रुग्णांना वित्तीय सहाय्य करण्यास इच्छुक असलेल्या व्यक्ती किंवा कॉर्पोरेट दाते यांना एकत्र आणण्यासाठी डिजीटल प्लॅटफॉर्म निर्माण करण्याचे प्रस्तावित आहे.

➤ **मनुष्यबळ विकास :-**

राज्यातील किमान एका वैद्यकीय महाविद्यालयात वैद्यकीय अनुवांशिकता विभाग (Department of Medical Genetics) निर्माण करण्याची विनंती राज्य शासनास करण्यात येईल. जेणेकरून

याबाबतचे शिक्षण देणे व वैद्यकीय व्यवसायिकांमध्ये या संदर्भातील जागृती वाढीस लागणे शक्य होईल.

जैव तंत्रज्ञान विभागांतर्गत स्थापन करण्यात आलेल्या निदान केंद्रांचा वापर हा वैद्यकीय व्यवसायिक व कर्मचारी यांना दुर्मिळ आजारांच्या तपासणी प्रशिक्षण देण्यासाठी सुध्दा करण्यात येईल.

- दुर्मिळ आजारांवरील उपचार, प्रतिबंध, संशोधन व विकास यासाठी राष्ट्रीय संस्थांचा संघ निर्माण करणे.
- दुर्मिळ आजारांवरील औषधे परवडणाऱ्या दरात मिळण्याच्या दृष्टीने प्रयत्न करणे.

०५. थोडक्यात दुर्मिळ आजारांवरील उपचारांसाठी आरोग्य व कुटुंब कल्याण मंत्रालयाने सर्वसमावेशक धोरण प्रस्तावित केले आहे. सदर धोरणामध्ये राज्य शासनाच्या स्तरावर पुढीलप्रमाणे कार्यवाही अपेक्षित दिसते.:-

गट-२ मधील दुर्मिळ आजारांच्या रुग्णांना सहाय्य देण्याबाबत राज्य शासनाने विचार करणे.

राज्यातील किमान एका वैद्यकीय महाविद्यालयात वैद्यकीय अनुवांशिकता विभाग (Department of Medical Genetics) निर्माण करणे.

०६. राज्यातील किमान एका वैद्यकीय महाविद्यालयात वैद्यकीय अनुवांशिकता विभाग (Department of Medical Genetics) निर्माण करण्याची शिफारस सदर धोरणात करण्यात आली आहे. तरी, सदर बाब वैद्यकीय शिक्षण व औषधी द्रव्ये विभागाच्या निदर्शनास आणून देण्यात येत असून त्या विभागास या संदर्भातील पुढील कार्यवाही करण्याची विनंती करण्यात येत आहे.

०७. गट-१ मधील दुर्मिळ आजार ज्यांवर एकदाच उपचार करणे आवश्यक आहे अशासाठी रु.२०.०० लाखापर्यंतचे वित्तीय सहाय्य राष्ट्रीय आरोग्य निधीमधून केंद्र शासनामार्फत उपलब्ध करून दिले जाणार आहे. राष्ट्रीय आरोग्य निधीबाबत सविस्तर मार्गदर्शक सूचना केंद्र शासनाने दिनांक ०१.०२.२०१९ च्या कार्यालयीन जापनान्वये प्रसिध्द केल्या आहेत.

०८. तरी, दुर्मिळ आजारावरील औषधोपचारसाठी राष्ट्रीय धोरण-२०२१ याला व्यापक प्रसिध्दी देण्यात यावी. सर्व शासकीय रुग्णालये, वैद्यकीय महाविद्यालयांशी संलग्न रुग्णालये, महानगरपालिका/ नगरपालिका/ जिल्हा परिषदांद्वारे संचालित केली जाणारी रुग्णालये येथे दुर्मिळ आजारांवरील उपचारांसाठी रुग्ण दाखल झाल्यास त्यांना उपरोक्त दोन्ही योजनांची माहिती देण्यात यावी.

०९. सदर शासन निर्णय महाराष्ट्र शासनाच्या www.maharashtra.gov.in या संकेतस्थळावर उपलब्ध करण्यात आला असून त्याचा संकेतांक २०२१०८१२११२३४०८३१७ असा आहे. हा शासन निर्णय डिजीटल स्वाक्षरीने साक्षांकित करून काढण्यात येत आहे.

महाराष्ट्राचे राज्यपाल यांच्या आदेशानुसार व नावाने.

LAHANE
VIJAYKUMAR
LAXMANRAO

Digitally signed by LAHANE VIJAYKUMAR LAXMANRAO
DN: c=IN, o=GOVERNMENT OF MAHARASHTRA,
ou=PLANNING DEPARTMENT, postalCode=400032,
st=Maharashtra,
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A6A7250,
serialNumber=8902AEDC74F3CACF3FB967FF5955AAF5D
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VIJAYKUMAR LAXMANRAO
Date: 2021.08.17 13:06:34 +05'30'

(वि. ल. लहाने)

सह सचिव, महाराष्ट्र शासन

प्रत:-

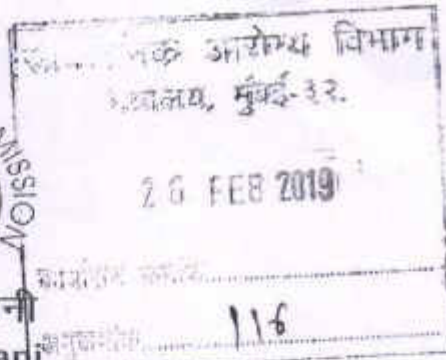
१. मा.राज्यपालांचे सचिव (५ प्रती) (पत्राने)
२. मा.मुख्यमंत्र्यांचे प्रधान सचिव (५ प्रती), मंत्रालय, मुंबई- ४०० ०३२.
३. सर्व मंत्री/ राज्यमंत्री यांचे खाजगी सचिव, मंत्रालय, मुंबई-४०० ०३२.
४. मा.विरोधी पक्षनेता, विधान परिषद/ विधान सभा, महाराष्ट्र विधानमंडळ सचिवालय, मुंबई.
५. सर्व सन्माननीय संसद सदस्य, विधानसभा व विधानपरिषद सदस्य.
६. मा.मुख्य सचिव यांचे वरिष्ठ स्वीय सहाय्यक, मंत्रालय, मुंबई-४०००३२.
७. प्रधान महालेखापाल (लेखा व अनुज्ञेयता)-१, महाराष्ट्र, मुंबई.
८. प्रधान महालेखापाल (लेखा व अनुज्ञेयता)-२, महाराष्ट्र, नागपूर.
९. प्रधान महालेखापाल (लेखापरीक्षा)-१, महाराष्ट्र, मुंबई.
१०. प्रधान महालेखापाल (लेखापरीक्षा)-२, महाराष्ट्र, नागपूर.
११. सर्व अपर मुख्य सचिव/ प्रधान सचिव/ सचिव, मंत्रालयीन प्रशासकीय विभाग,
१२. महाप्रबंधक, मा.मुंबई उच्च न्यायालय, मुंबई.
१३. प्रबंधक, मुंबई उच्च न्यायालय (मूळ शाखा), मुंबई.
१४. प्रबंधक, मा.लोकायुक्त व मा.उप लोकायुक्त यांचे कार्यालय, नवीन प्रशासन भवन, हुतात्मा राजगुरु चौक, मंत्रालय, मुंबई.
१५. प्रधान सचिव, विधानमंडळ सचिवालय, विधान भवन, मुंबई.

१६. मा.मुख्य माहिती आयुक्त, राज्य माहिती आयोग, मुंबई.
१७. सर्व विभागीय आयुक्त
१८. आयुक्त (आरोग्य सेवा) तथा अभियान संचालक, राष्ट्रीय आरोग्य अभियान, मुंबई.
१९. प्रकल्प संचालक, महाराष्ट्र राज्य एड्स नियंत्रण संस्था, वडाळा, मुंबई.
२०. मुख्य कार्यकारी अधिकारी, महाराष्ट्र राज्य कामगार विमा सोसायटी, पंचदीप भवन, ६ वा मजला, ना.म.जोशी मार्ग, लोअर परेल, मुंबई- ४०००१३.
२१. मुख्य कार्यकारी अधिकारी, राज्य आरोग्य हमी सोसायटी, जीवनदायी भवन, राज्य कामगार विमा रुग्णालय आवार, गणपत जाधव मार्ग, वरली, मुंबई- ४०००१८.
२२. संचालक आयुष, आयुष संचालनालय, मुंबई.
२३. सर्व महानगरपालिका आयुक्त.
२४. सर्व जिल्हाधिकारी,
२५. संचालक, नगरपरिषद प्रशासन संचालनालय, वरली, मुंबई.
२६. सर्व जिल्हा परिषदांचे मुख्य कार्यकारी अधिकारी
२७. संचालक (आरोग्य सेवा-१), आरोग्य सेवा आयुक्तालय, मुंबई.
२८. संचालक (आरोग्य सेवा-२), आरोग्य सेवा संचालनालय, पुणे.
२९. अतिरिक्त अभियान संचालक, राष्ट्रीय नागरी आरोग्य अभियान, मुंबई.
३०. अतिरिक्त संचालक, आरोग्य सेवा, कुटुंब कल्याण, माताबाल संगोपन व शालेय आरोग्य, पुणे.
३१. उप संचालक, सर्व मंडळ कार्यालये.
३२. सर्व जिल्हा शल्य चिकित्सक (FWB मार्फत)
३३. सर्व जिल्हा आरोग्य अधिकारी, (FWB मार्फत)
३४. महासंचालक, माहिती व जनसंपर्क महासंचालनालय, मुंबई (५ प्रती)
३५. भारतीय जनता पार्टी, महाराष्ट्र प्रदेश, सी.डी.ओ.बॅरेक नं.१, योगक्षेम समोर, वसंतराव भागवत चौक, नरिमन पॉईंट, मुंबई-४०००२०.
३६. इंडियन नॅशनल काँग्रेस, महाराष्ट्र प्रदेश काँग्रेस (आय) समिती, टिळक भवन, काकासाहेब गाडगीळ मार्ग, दादर, मुंबई-४०००२५.
३७. राष्ट्रवादी काँग्रेस पार्टी, राष्ट्रपती भवन, फ्री प्रेस जर्नल मार्ग, नरिमन पॉईंट, मुंबई-४०००२१.
३८. शिवसेना, शिवसेना भवन, गडकरी चौक, दादर, मुंबई-४००००१.
३९. बहुजन समाज पार्टी, डी-१ इन्सा हटमेंट, आझाद मैदान, मुंबई-४००००१.
४०. भारतीय कम्युनिस्ट पार्टी, महाराष्ट्र कमिटी, ३१४, राजभवन, एस.व्ही.पटेल रोड, मुंबई.
४१. भारतीय कम्युनिस्ट (मार्क्सवादी) पार्टी, महाराष्ट्र कमिटी, जनशक्ती हॉल, ग्लोब मिल पॅलेस, वरली, मुंबई.
४२. सार्वजनिक आरोग्य विभागातील सर्व कार्यासने, मंत्रालय, मुंबई-४००००१.
४३. निवडनस्ती (कार्यासन आरोग्य-७)



मनोज झालानी
Manoj Jhalani

अपर सचिव एवं मिशन निदेशक (रा.स्वा.मि.)
Additional Secretary & Mission Director (NHM)



स्वा.पद - (११)

भारत सरकार

स्वास्थ्य एवं परिवार कल्याण मंत्रालय
निर्माण भवन, नई दिल्ली - 110011

GOVERNMENT OF INDIA
MINISTRY OF HEALTH & FAMILY WELFARE
NIRMAN BHAVAN, NEW DELHI - 110011

D.O. No.: C-171011/1/2018-RD

Dated the 15th February, 2019

Subject: - Umbrella Scheme of Rashtriya Arogya Nidhi (RAN) - Sub Component Scheme for providing one-time financial assistance to persons below threshold poverty line for one-time treatment of specified rare diseases - regarding.

Dear colleague,

The Ministry of Health and Family Welfare has constituted a committee under the chairmanship of DGHS to formulate a revised Policy on rare diseases as the erstwhile National Policy for Treatment of Rare Diseases, 2017 has been kept in abeyance vide Gazette Notification dated 18.12.2018. Ad-interim, Government has also approved a Sub component Scheme for providing one-time financial assistance to persons below threshold poverty line for one-time treatment of specified identified rare diseases under the new Umbrella Scheme of Rashtriya Arogya Nidhi (RAN). Details of the diseases approved for one-time treatment, application form etc. may be viewed in the RAN guidelines uploaded on this Ministry's website www.mohfw.gov.in under link: *What's New - Disease Alerts - Rare Diseases*.

2. The Government has also approved a budget provision of Rs.7.5 crores for treatment of the specified rare diseases under the above mentioned umbrella scheme of RAN for the current financial year. An amount of Rs.200 crores has also been budgeted for the Umbrella Scheme of RAN in 2019-2020.

3. One-time financial assistance for one-time treatment of specified rare diseases is a new component under the umbrella scheme of RAN. Therefore, you are requested to give wide publicity of the new scheme for the benefit of the masses, Doctors, Hospitals, ASHAs, Caregivers, other health workers and Research and Referral Laboratories etc. in your State so that the eligible patients may avail the benefits of the new Scheme. A copy of the approved guidelines of the Umbrella Scheme of RAN is also attached herewith for necessary action at your end.

With regards,

Yours faithfully,

(Manoj Jhalani)

Encl: as above

Additional Chief Secretary / Principal Secretary / Secretary (H&FW) of all States/UTs

स्वच्छ भारत-स्वस्थ भारत

Telefax : 23063687 23063693 E-mail : manoj.jhalani@nic.in

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03/19

No. W.11036/39/2017-RAN
Government of India
Ministry of Health and Family Welfare
Department of Health & Family Welfare

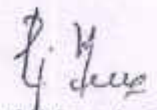
New Delhi, dated the 1st February, 2019.

Office Memorandum

Subject: Guidelines regarding implementation of Umbrella Scheme of Rashtriya Arogya Nidhi (RAN).

The undersigned is directed to say that **Rashtriya Arogya Nidhi (RAN) Society** was set up in 1997 to provide financial assistance to patients, living below poverty line and who are suffering from major life threatening diseases, to receive medical treatment at any of the super specialty Government hospitals / institutes. In its meeting held on 7.8.2018, the Managing Committee of the RAN Society has decided, inter alia, that the Society shall be closed w.e.f. 1.1.2019. Accordingly the function of the RAN Society will now vest in the Department of Health and Family Welfare w.e.f. 1.1.2019 and RAN scheme including Health Minister's Cancer Patients Fund will be implemented by the Department of Health and Family Welfare.

2. To provide financial assistance to poor patients a new Umbrella Scheme of Rashtriya Arogya Nidhi has been formulated with the approval of the competent authority. The Umbrella Scheme of Rashtriya Arogya Nidhi (RAN) will have three components namely (i) Rashtriya Arogya Nidhi (RAN), (ii) Health Minister's Cancer Patients Fund (HMCPF) and (iii) Scheme for financial assistance for patients suffering from specified rare diseases. A copy of guidelines of the new Umbrella Scheme is enclosed for information and necessary action.


(R.J. Meena)

Under Secretary to the Government of India
Tel: 23061986

To

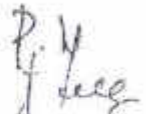
1. All the Government hospitals (as per list enclosed).
2. All Regional Cancer Centres (as per list enclosed).
3. All State Cancer Institutes and Tertiary Cancer Care Centres (As per list enclosed).
4. Health Secretaries of all State Governments/UT Administrations.

Copy to:

1. Finance Division.
2. Under Secretary (PH/RD).
3. Director (Budget).

Copy for information to :

1. PS to HFM.
2. PS to MoS (AKC).
3. PS to MoS (AP).
4. Sr. PPS to Secretary (HFW).
5. Sr. PS to AS&FA.
6. Sr. PS to AS (Health).
7. Sr. PS to AS&MD.
8. PS to Addl. DG (AKG).
9. Sr. PS to JS (Rare Diseases).
10. Sr. PS to EA (NS).
11. PS to CCA.
12. Addl. DDG (BRS).


(R.J. Meena)

Under Secretary to the Government of India
Tel: 23061986

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Guidelines regarding implementation of Umbrella Scheme of Rashtriya Arogya Nidhi (RAN).

The Umbrella Scheme of Rashtriya Arogya Nidhi (RAN) will have three components namely (i) Rashtriya Arogya Nidhi (RAN), (ii) Health Minister's Cancer Patients Fund (HMC PF) and (iii) Scheme for financial assistance for patients suffering from specified rare diseases.

2. The objectives of the three components of the RAN Scheme are given below:

- (a) **Rashtriya Arogya Nidhi**— to provide financial assistance to poor patients living below threshold poverty line and suffering from life threatening diseases relating to heart, kidney, liver, etc for their treatment at Government hospitals/institutes having super specialty facilities.
- (b) **Health Minister's Cancer Patient Fund** — to provide financial assistance to poor patients living below threshold poverty line and suffering from cancer, for their treatment at Regional Cancer Centres (RCCs)/Tertiary Care Cancer Centres (TCCC) and State Cancer Institutes (SCI).
- (c) **Financial assistance Scheme for poor patients suffering from rare diseases** — to provide financial assistance to poor patients living below threshold poverty line and suffering from specified rare diseases for their treatment at Government hospitals/institutes having super specialty facilities.

3. Details of guidelines and procedures for implementation of the Umbrella Scheme of RAN are as under.

4. General conditions governing the Umbrella Scheme of RAN are as under:

- (i) Financial assistance will be provided to poor patients living below State/UT-wise threshold poverty line as per **Annexure-I**.
- (ii) List of diseases covered for financial assistance under the Umbrella Scheme of RAN is at **Annexure-II**.
- (iii) Patients will be provided financial assistance for their treatment at Government hospitals having super specialty facilities. Those having treatment in private hospitals will not be eligible for financial assistance under the scheme.
- (iv) The financial assistance to eligible patients will be in the form of 'one-time grant'.
- (v) Government servants and their families will not be eligible under this scheme.
- (vi) There will be no reimbursement of expenditure already incurred.

- (vii) Families covered under Ayushman Bharat – Pradhan Mantri Jan Arogya Jojna (PMJAY) will not be eligible for financial assistance under RAN and HMCPF components.
- (viii) In a bid to speed up the assistance to the needy patients, Revolving Funds are set up in Government Hospitals/Institutes listed at **Annexure-III**. Funds up to Rs. one crore for each component separately (Rs. two crore in case of AIIMS, New Delhi for RAN component only) will be placed at their disposal.
- (ix) Powers are delegated to the Medical Superintendent/Director of the hospitals with revolving funds for providing treatment up to Rs. 5 lakh for eligible patients in each case, out of revolving fund.
- (x) More hospitals shall be added in the list of hospitals with revolving fund as and when such hospitals are identified by the Technical Committee.
- (xi) Cases involving treatment beyond Rs. 5 lakh in hospitals with revolving fund and all the cases for financial assistance from hospitals not having revolving fund will be referred to Department of Health & Family Welfare, Government of India for approval.
- (xii) Funds shall be released to hospitals not having revolving fund in respect of cases approved by the Department of Health and Family Welfare along with the sanction letter.
- (xiii) No separate funds will, however, be released to hospitals having revolving funds in respect of cases received in and approved by Department of Health and Family Welfare, involving treatment beyond 5 lakh and such expenditure will be met out of the revolving funds.
- (xiv) In case of hospitals having revolving fund, the cost of treatment in all eligible cases will be met out of the revolving fund, which will be replenished from time to time, on the basis of utilization certificate and list of beneficiaries furnished by the hospitals. On utilization of 75% of the amount placed in revolving fund, hospitals will become eligible for replenishment.
- (xv) Maximum financial assistance admissible under the Scheme will be Rs. 15 lakh.
- (xvi) Financial assistance received under Prime Minister's National Relief Fund (PMNRF) or from any other source by the patient for treatment, shall be deducted from the admissible amount of financial assistance under the Umbrella Scheme of RAN.
- (xvii) For RAN and HMCPF components, there will be a Technical Committee, which shall advise the Government in technical matters such as enlisting of diseases covered under Umbrella Scheme of RAN and identifying Government hospitals/institutes for setting up of revolving funds, examining requests for financial assistance received in the Department and giving their

P. N. K.

recommendations on any other matter of technical nature. The composition of the Technical Committee will be as under:

1. Addl. DG/Adviser, DGHS.
2. Joint Secretary/Economic Adviser concerned.
3. Medical Superintendent, Dr. RML Hospital, New Delhi.
4. HOD, Cardiology, AIIMS, New Delhi.
5. HOD, Haematology, AIIMS, New Delhi.
6. HOD, Medical Oncology, Safdarjung Hospital, New Delhi

(xviii) Experts in rare diseases will be associated with the above Technical Committee to advise the Government in matter listed at sub para xvii above, in respect of rare diseases.

5. **Conditions specific to Scheme for financial assistance for patients suffering from specified rare diseases**

(i) The objective of this component is to provide financial assistance to poor patients living below State/UT-wise threshold poverty line (**Annexure-I**) and suffering from specified Rare Diseases (**Annexure-II**), to begin with, for their treatment at Government hospitals having super specialty facilities.

(ii) The rare diseases specified in **Annexure-II** are disorders identified based on availability of treatment, reasonably proven clinical outcomes and cost effectiveness. The list of specified rare diseases for financial assistance under Rare Diseases component of Umbrella Scheme of RAN will be as suggested by the relevant Technical Committee.

(iii) Patients suffering from rare diseases as specified in **Annexure-II** belonging to the socio-economic categories that are eligible to be PMJAY beneficiaries will also be considered for packages not covered under PMJAY.

6. Common application form has been prescribed (**Annexure-IV**) for submission of request for financial assistance under any of the component of the Umbrella Scheme of RAN. The application shall be signed by the treating doctor/Head of the Department and countersigned by the Medical Superintendent of the hospital concerned.

7. Application for financial assistance shall be submitted along with:

(i) Income certificate (in original) of the patient/parents/other major earning members of the family, indicating income from all sources, duly certified by the Block/Mandal Development Officer/Tehsildar/SDM/ Administrator/Special Officer of Municipal Boards/District Officer.

(ii) A copy of the entire ration card along with its cover page, with details of all the family members, issued by the Food and Civil Supplies Department of the State/UT Government duly self attested.

8. All Government hospitals, where revolving funds are set up under RAN as well as Rare Diseases components, will open separate bank accounts for the two sub components and submit the

bank details to the Ministry immediately so that funds could be transferred to them under the Umbrella Scheme of RAN.

9. Similarly all 27 Regional Cancer Centres, where revolving funds are set up under HMCPF component, will open a separate bank account for the purpose and submit the bank details to the Ministry immediately so that funds could be transferred to them under the Umbrella Scheme of RAN.

10. This has approval of the competent authority.

Enc: Annexure I to IV.

P. W. Lee

State-wise poverty line for Rural and Urban Areas w.e.f. 1/1/2019

In Rupees

State/UT	Per capita per month poverty line as fixed by erstwhile Planning Commission for 2011-12		Revised per capita threshold income per month w.e.f. 1.1.2019 under Umbrella Scheme of Rashtriya Agya Nidhi	
	Rural	Urban	Rural	Urban
ALL India	816	1000	1160	1388
Andhra Pradesh	860	1009	1191	1529
Arunachal Pradesh	930	1060	1495	1701
Assam	828	1008	1190	1387
Bihar	778	923	1134	1269
Chhattisgarh	738	849	1064	1185
Delhi	1145	1134	1571	1605
Goa	1090	1134	1668	1522
Gujarat	932	1152	1327	1530
Haryana	1015	1169	1902	1593
Himachal Pradesh	913	1064	1239	1467
J & K	891	988	1339	1397
Jharkhand	748	974	1095	1349
Karnataka	902	1089	1294	1578
Kerala	1018	987	1484	1425
Maharashtra	967	1126	1388	1484
Madhya Pradesh	771	897	1047	1258
Manipur	1118	1170	1850	1629
Meghalaya	888	1154	1248	1536
Mizoram	1066	1155	1488	1500
Nagaland	1270	1302	1976	1808
Odisha	695	861	1011	1180
Puducherry	1301	1309	1881	1838
Punjab	1054	1155	1475	1563
Rajasthan	905	1002	1272	1411
Sikkim	930	1226	1407	1808
Tamil Nadu	880	937	1266	1331
Telangana	860	1009	1264	1395
Tripura	798	920	1191	1334
Uttar Pradesh	768	941	1061	1327
Uttarakhand	880	1082	1208	1475
West Bengal	783	981	1138	1368

For Andaman Nicobar Islands use threshold levels of Tamil Nadu, for Dadra and Nagar Haveli and for Daman and Diu use levels for Gujarat, and for Lakshadweep use levels for Kerala, for Chandigarh use levels for Punjab.

Illustrative list of categories of treatment for financial assistance under RAN component

(This list is reviewed by the Technical Committee from time to time)

1. Cardiology & Cardiac Surgery:

- 1) Pacemakers including CRT/Biventricular pacemaker.
- 2) Automatic Implantable Cardioverter Defibrillator (AICD) and Combo devices.
- 3) Coronary Artery Disease including Diagnostic Cardiac Catheterization and Coronary Angiography.
- 4) Interventional procedure including Angioplasty, with or without Stents (Bare metal Stents as well as Drug Eluting Stents) Rota-ablation, Balloon Valvuloplasty.
- 5) ASD, VSD and PDA device closure.
- 6) Peripheral Vascular Angioplasty including Carotid Angioplasty & Renal Angioplasty, Aortic Surgery and Stent Grafting.
- 7) Coil Embolization and Vascular plugs.
- 8) Electrophysiological Studies (EPS) and Radio Frequency (RF) Ablation.
- 9) Cardio vascular surgery for Congenital and Acquired conditions including C.A.B.G, Valve replacement etc.
- 10) Heart/Lung Transplantation. (ceiling cost may be upto CGHS rates)
- 11) Intra Aortic Balloon Pump (IABP).
- 12) Thrombolytic Therapy for Acute Myocardial Infarction, Pulmonary Thromboembolism & Prosthetic valve Thrombosis.
- 13) IVC Filter

2. Cancer :

- 1) Radiation treatment of all kinds including Radio Therapy and Gama Knife Surgery/GRT/MRT/Brachytherapy.
- 2) Anti-Cancer Chemotherapy with supportive medication including hormonal therapy.
- 3) Bone Marrow Transplantation- Allogenic & Autologous
- 4) Diagnostic Procedures- including PET scan.
- 5) Surgery for operable malignant tumours.

3. Urology/Nephrology/Gastroenterology/GI Surgery :

- 1) Dialysis (Both Haemodialysis as well as Peritoneal)
- 2) Plasmapheresis in ABO incompatible donors
- 3) Continuous RRT in Acute Renal failure
- 4) Vascular access consumables (AV Grafts, Catheters including perm catheters) for Dialysis.
- 5) Renal transplant- The ceiling cost may be upto CGHS rates.
- 6) USG guided PCNL and USG guided SPC.
- 7) Endoscopic surgical procedures in urology including CPE with TURBT, CPE with endoscopic catheterization, CPE with clot evacuation.
- 8) Endoscopic surgical procedures in GI surgery.
- 9) Acute GI emergencies like Acute Pancreatitis, GI bleeding, Cholangitis, Peritonitis, Intestinal Obstruction, Biliary stricture, Acute Fulminant Hepatitis, Hepatic Encephalopathy, Hepatic Abscess etc.
- 10) Liver Transplantation and Surgery for portal hypertension- the ceiling cost may be upto CGHS rates.

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4. Orthopedics:

- 1) Treatment of Traumatic & Pathological Fracture.*
- 2) Implants for joint replacement.**
- 3) Spinal fixation Implant.**

*Only Indigenous implants for use in Fracture and Poly Trauma will be permitted under the scheme.

**Justification for Non Indigenous Implants would be provided by the treating doctor.

5. Neurosurgery – Neurology :

- 1) Brain Tumours
- 2) Head injury
- 3) Intracranial aneurysms & aneurysms of neck vessels.
- 4) Vascular Malformations of brain & spinal cord
- 5) Spinal Tumors
- 6) Degenerative /Demyelinating diseases of brain/spinal cord
- 7) Cerebrospinal Stroke
- 8) Status Epileptics
- 9) Movement Disorders
- 10) Neurological infections (Acute/chronic)
- 11) Traumatic Spinal Injury
- 12) Occlusive Vascular Disease of Brain
- 13) Guillain-Barre syndrome
- 14) Myasthenia Gravis in Crisis (medical and surgical)
- 15) Acute Polymyositis with Ventilatory Failure
- 16) Acute or Chronic Autoimmune Diseases

6. Endocrinology :

- 1) Complications and sequel of diabetes which require one time treatment e.g. Amputation or Renal Transplant or Retinal detachment, Glaucoma etc..
- 2) GH deficiency
- 3) Adrenal insufficiency (Acute/Chronic)
- 4) Cushing's Disease
- 5) Endocrine surgery with post surgical treatment for one year.
- 6) Metabolic Bone Disease/Renal Tubular Acidosis.

7. Mental Illness :

Any treatment requiring one time grant for mental disorders including

- 1) Organic Psychosis (acute/ chronic).
- 2) Functional psychosis including Schizophrenia, Bio-polar Disorders, delusional disorders and other acute polymorphic psychosis
- 3) Severe OCD, Somatoform disorders.
- 4) Developmental disorders including Autism spectrum disorders and Severe behavioral disorders during childhood.
- 5) Psycho diagnostics, neuropsychological assessments, IQ assessment, blood tests like serum lithium and drug level of carbamazepine, valproate, phenytoin and any other similar medications.
- 6) CSF studies screening for substances abuse/toxicology.

8. Gynecology :

Uterine Artery Embolization for Post Partum Hemorrhage

9. Rare Diseases :

1. Disorders amenable to treatment with Hematopoietic Stem Cell Transplantation (HSCT)

- i. Lysosomal storage disorders in early stages for which Enzyme replacement Therapy (ERT) is presently not available (eg Metachromatic Leukodystrophy, Krabbe's disease and severe form of Mucopolysaccharoidosis (MPS) type I within first 2 years of age.
- ii. Adrenoleukodystrophy (early stages), before the onset of hard neurological signs.
- iii. Immune deficiency disorders eg Severe Combined Immunodeficiency (SCID), Chronic Granulomatous disease, Wiskot Aldrich Syndrome, etc
- iv. Osteopetrosis
- v. Fanconi Anemia
- vi. Others to be decided on case to case basis

2. Disorders amenable to organ transplantation

- i. Liver Transplantation -Metabolic Liver diseases and other Inborn Errors of Metabolism (small molecule diseases)
 - i. Tyrosinemia (failure to respond to nitisinone therapy or have documented evidence of malignant changes in hepatic tissue)
 - ii. Glycogen storage disorders (GSD) I, III and IV due to poor metabolic control, multiple liver adenomas, or high risk for Hepatocellular carcinoma or evidence of substantial cirrhosis or liver dysfunction or progressive liver failure,
 - iii. MSUD (Maple Syrup Urine Disease),
 - iv. Urea cycle disorders,
 - v. Organic acidemias,
 - vi. Wilson's disease (Decompensated cirrhosis)
 - vii. Bile acid synthetic defects (Decompensated cirrhosis)
 - viii. CriglerNajjar Type 1,
 - ix. Alpha 1 antitrypsin deficiency (Decompensated cirrhosis)
 - x. Progressive familial Intrahepatic Cholestasis (PFIC)(Decompensated cirrhosis)
 - xi. Others to be decided - case to case basis
- ii. Renal Transplantation-
 - i. Fabry's disease,
 - ii. Autosomal recessive Polycystic Kidney Disease (ARPKD),
 - iii. Autosomal dominant Polycystic Kidney Disease (ADPKD)
 - iv. Others on case to case basis

iii) Patients requiring combined liver and kidney transplants -Rarely Methyl Malonic aciduria may require combined liver & Kidney transplant) etc

10. Miscellaneous :

Any life saving procedure/ any other major illness/treatment/intervention not covered under any of the components of Umbrella Scheme of RAN and recommended by the Technical Committee could be considered for financial assistance .

J. K. Singh

(A) List of hospitals/institutes, where revolving funds are set up under RAN and Rare Diseases components of the Umbrella Scheme of RAN

1. AIIMS, New Delhi
2. Dr. RML Hospital, New Delhi
3. Safdarjung Hospital, New Delhi.
4. Lady Hardinge Medical College & Smt. SK Hospital, New Delhi.
5. PGI, Chandigarh
6. JIPMER, Pudducherry
7. NIMHANS, Bangalore
8. SGPGIMS, Lucknow
9. CNCI, Kolkata
10. KGMC, Lucknow
11. NEIGRIHMS, Shillong
12. RIMS, Imphal.
13. SKIMS, Srinagar.
14. Sree Chitra Tirunal Institute of Medical Sciences and Technology, Thiruvananthapuram.

(B) List of 27 Regional Cancer Centres, where revolving fund are set under HMCPF component

1. Kamala Nehru Memorial Hospital, Allahabad, Uttar Pradesh.
2. Chittaranjan National Cancer Institute, Kolkata, West Bengal
3. Kidwai Memorial Institute of Oncology, Bangalore, Karnataka. (SCI)
4. Regional Cancer Institute (WIA), Adyar, Chennai, Tamil Nadu. (SCI)
5. Acharya Harihar Regional Cancer, Centre for Cancer Research & Treatment, Cuttack, Orissa. (SCI)
6. Regional Cancer Control Society, Shimla, Himachal Pradesh.
7. Cancer Hospital & Research Centre, Gwalior, Madhya Pradesh.
8. Dr. B.R.A. Indian Rotary Cancer Institute, (AIIMS), New Delhi.
9. R.S.T. Hospital & Research Centre, Nagpur, Maharashtra. (TCCC)
10. Pt. J.N.M. Medical College, Raipur, Chhatisgarh.
11. Post Graduate Institute of Medical Education & Research (PGIMER), Chandigarh.
12. Sher-I- Kashmir Institute of Medical Sciences, Soura, Srinagar.
13. Regional Institute of Medical Sciences, Manipur, Imphal.
14. Govt. Medical College & Associated Hospital, Bakshi Nagar, Jammu.
15. Regional Cancer Centre, Thiruvananthapuram, Kerala
16. Gujarat Cancer Research Institute, Ahmadabad, Gujarat.
17. MNJ Institute of Oncology, Hyderabad, Andhra Pradesh. (SCI)
18. Pondicherry Regional Cancer Society, JIPMER, Pondicherry.
19. Dr. B.B. Cancer Institute, Guwahati, Assam.
20. Tata Memorial Hospital, Mumbai, Maharashtra.
21. Indira Gandhi Institute of Medical Sciences, Patna, Bihar. (SCI)
22. Acharya Tulsi Regional Cancer Trust & Research Institute (RCC), Bikaner, Rajasthan.
23. Regional Cancer Centre, Pt. B.D. Sharma Post Graduate Institute of Medical Sciences, Rohtak, Haryana.
24. Civil Hospital, Aizawl, Mizoram. (TCCC)
25. Sanjay Gandhi Post Graduate Institute of Medical Sciences, Lucknow. (TCCC)
26. Government Arignar Anna Memorial Cancer Hospital, Kancheepuram, Tamil Nadu.
27. Cancer Hospital, Tripura, Agartala. (SCI)

P. Y. Lux

Application for Financial Assistance under Umbrella Scheme Rashtriya Arogya Nidhi (RAN) and Health Minister's Discretionary Grant (HMDG)

(Please tick mark (/))

Rashtriya Arogya Nidhi		HEALTH MINISTER'S DISCRETIONARY GRANTS	
Health Minister's Cancer Patient Fund			
Rare Diseases			

1	Name of the Patient (in Block Letters)	
2	Age	
3	(a) Permanent Address along with Pin Code	
	(b) Address for correspondence	
4	(a) Email Address (if available)	
	(b) Mobile No. (if available)	
5	(a) Father's /Mother's name	
	(b) Husband/wife's name	
6	Applicant's Relationship with the Patient	
7	Disease from which suffering (Name of the disease)	
8	Whether the applicant or the person on whom the patient is dependent, is an employee of Centre/State Government /Pensioner	
9	Monthly Income of the applicant and all family members from all sources issued by Tehsildar/BDO/SDO/SDM/DC. (Original Income Certificate should be attached. However, where online certificates are issued, self attested copy of income certificate may be enclosed)	
10	Amount of Financial Assistance required	
11	Whether financial assistance has been received from (a) any Ministry/Department other than Ministry of Health & Family Welfare such as Prime Minister National Relief Fund or CM Relief Fund for treatment of the same disease.	
	(b) Ministry of Health & Family Welfare earlier. If so, full details may be given.	
12	Attach self attested copy of the Ration Card	

P. V. K.

13	<p>Aadhar Card No., if any (Attach self attested copy)</p> <p>I hereby share my Aadhaar Number issued by UIDAI & voluntarily give my consent to link my Aadhaar Number with my request for financial assistance under RAN/HMCPF/Rare Disease/HMDG Schemes. I also authorize Ministry of Health & Family Welfare to use my Aadhaar card details & identity information for authentication with UIDAI.</p>	26/9
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DECLARATION

1. I declare that the information given above is correct and complete in all respect.
2. I am not covered for benefits under Pradhan Mantri Jan Arogya Yojna (PMJAY).

OR

(For rare diseases)

Though I am covered under PMJAY, the amount of financial assistance required, indicated at Column 10 above, is only for packages not covered under PMJAY.

Date :

Signature of the Applicant/Patient

TO BE FILLED BY THE M.O. INCHARGE OF THE CASE/HOSPITAL, ETC, WHERE THE PATIENT IS RECEIVING TREATMENT

1. Name of the Patient & Hospital Registration No. _____
2. Gist of Reports of important Investigations done _____
3. Diagnosis-A short Note on the present clinical conditions may be indicated _____
4. If the patient has been operated, please Indicate the date of operation _____
- 5.(a)The name of the Hospital where the patient is receiving treatment. _____
- (b) Whether Hospital is Government or Private. _____
6. Amount recommended for treatment _____
7. Probable date of operation/intervention _____

P. H. K.

8. Item wise break up of expenditure recommended in Column 6

Name of consumables/medicines required for operation/treatment	Cost (In Rupees)
1.	
2.	
3.	
4.	
5.	

9. Certified that the patient is not covered for benefits under PMJAY.

OR

(For rare diseases)

For patients covered under PMJAY, the amount recommended for treatment under Column 6 above, is only for packages not covered under PMJAY.

Signature of the HOD/MO-in-charge
(Note below the level of Consultant/Assistant Professor with Official Seal)

1. Certified that the patient's particulars given above are true to the best of my knowledge and belief.
2. Details of Bank account (including IFSC Code) of the hospital to which funds are to be transferred (Bank details are to be provided in cases of hospitals, where revolving funds have not been set up).

Name of the Bank & Address	
Account No.	
IFSC code	
PAN/TIN/TAN	
Details of the account holder- name of hospital/institute	
Mobile Number	
E-mail id	

Signature of the Medical Superintendent of the
Hospital/Medical Institution with Official Seal

[Handwritten Signature] -12-

सहपत्र - (2)

26/11

No. W.11036/39/2018-RAN
Government of India
Ministry of Health and Family Welfare
Department of Health & Family Welfare

New Delhi, dated the 30th October, 2019.

Office Memorandum

Subject: Guidelines regarding implementation of Umbrella Scheme of Rashtriya Arogya Nidhi (RAN)-Clarification regarding.

The undersigned is directed to refer to this Ministry's OM No. No. W.11036/39/2017-RAN dated 1.2.2019 vide which guidelines regarding implementation of Umbrella Scheme of Rashtriya Arogya Nidhi (RAN) were circulated. The guidelines dated 1.2.2019 provides, inter alia, that families covered under Ayushman Bharat – Pradhan Mantri Jan Arogya Jojna (PMJAY) will not be eligible for financial assistance under RAN and HMCPF components.

2. During implementation of the RAN scheme, certain instances of PMJAY beneficiaries have been encountered, which are clarified as under::

- (i) Subject to fulfilment of eligibility, financial assistance shall be admissible under RAN and HMCPF components of the Umbrella Scheme of Rashtriya Arogya Nidhi to the patients from States/UTs which are not implementing the AB-PMJAY and they are having treatment in Government hospital, either in their own State/UT or outside, till such time that PMJAY is not implemented by the State/UT concerned.
- (ii) Except case as indicated in (i) above, patients eligible for treatment under AB-PMJAY shall not be eligible for financial assistance under the RAN and HMCPF component of the RAN scheme on any ground.
- (iii) In all the cases, the treating hospitals shall invariably certify as to whether the patient is covered under AB-PMJAY as per SECC database or not. Presently, the certificate is required to be given by the treating hospital in the application form itself by ticking the relevant option. However, few hospitals are not furnishing such certificate while forwarding the application form for financial assistance or the certificate is vague. It has now been decided to prescribe a separate certificate which should be furnished by the treating Government hospital while forwarding the application for financial assistance. Format of certificate is attached.

All concerned State/UT Governments and Government hospitals concerned are, therefore, requested to ensure compliance of the above decisions so that timely financial assistance could be provided to eligible poor patients.

Enc: As above.

(Kumar Pranav)
Under Secretary to the Government of India
Tel: 23061881

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4855
SPN 4575
5/11/19
- 3095
7/11
21/11/2019
- 26.11.2019
25.11
1. All the Government hospitals (as per list enclosed).
 2. All Regional Cancer Centres (as per list enclosed).
 3. All State Cancer Institutes and Tertiary Cancer Care Centres (As per list enclosed).
 4. Health Secretaries of all State Governments/UT Administrations.
- Important
To
PE Sh
hy
5/11/2019
DS(B)

CERTIFICATE

1. Certified that _____ (Name of the patient) is not covered for benefits under Pradhan Mantri Jan Arogya Yojna (PMJAY) as per SECC data base.

OR

(For rare diseases)

2. Though _____ (Name of the patient) is covered under PMJAY as per SECC database, the amount of financial assistance required, as indicated at Column 10 of the Application form, is only for packages not covered under AB-PMJAY.

Signature of the HOD/MO-in-charge

(Note below the level of Consultant/Assistant Professor with Official Seal)

साहजल - (3)

Dated 30th March, 2021

Subject: National Policy for Rare Diseases-regarding.

2. However, owing to implementation challenges, the said policy was kept in abeyance till the revised policy was issued or till further orders, whichever was earlier. A gazette notification informing the general public to this effect was also issued on 18.12.2018. Given the challenges in implementing the policy, the need for wider consultation and recommendations, a decision was taken to reframe the National Policy for Rare Diseases. Accordingly, ... Expert Committee was constituted by Ministry of Health and Family Welfare in November, 2018 to review the NPTRD, 2017 and draft Policy for Rare Diseases.

3. Based on the report of the Expert Committee and with the approval of the Competent Authority, a draft of the National Policy for Rare Diseases had been finalized and placed in the public domain *vide* this Ministry's Public Notice No. W.11037/1/2020-Grants(RD) dated 13.01.2020, with a view to elicit comments/views of the stakeholders, including the general public.

4. In view of the comments/views received and with the approval of the Competent Authority, National Policy for Rare Diseases, 2021 has been finalized and is available on the website of this Ministry (URL: <https://main.mohfw.gov.in/documents/policy> and <https://main.mohfw.gov.in/media/news-and-highlights>). A copy of the said policy is enclosed herewith for ready reference.

30/03/2021

(Rajeev Kumar)

Under Secretary to the Government of India

Tele: 011-23063019

To,

1. The Secretary, Department of Biotechnology, Ministry of Science and Technology, Government of India;
2. The Secretary, Department of Health Research/Director General, Indian Council for Medical Research, New Delhi;

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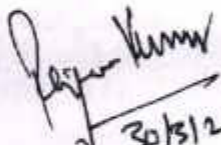
3. The Secretary, Department of Pharmaceuticals, Shashtri Bhawan, New Delhi;
4. The Secretary, Department for Promotion of Industry and Internal Trade, Udyog Bhawan, New Delhi;
5. The Secretary, Department of Revenue, Ministry of Finance, Government of India;
6. The Chairman, National Pharmaceutical Pricing Authority, 3rd/5th Floor, YMCA Cultural Center Building 1, Jai Singh Road, New Delhi;
7. The Drugs Controller General of India, FDA Bhawan, New Delhi;
8. Director/In-charge, Centre of Excellences (as per list enclosed);
9. Additional Chief Secretaries/Principal Secretaries/Commissioner (Health) of all States Governments/UT Administration (as per list enclosed).

Copy to:

1. Finance Division;
2. Director (Budget).

Copy for information to:

1. PS to HFM;
2. PS to MoS (AKC);
3. Sr. PPS to Secretary (HFW);
4. Sr. PPS to DGHS, Dte. GHS;
5. PPS to AS & FA;
6. Sr. PPS to AS & MD;
7. Sr. PPS to AS (Health);
8. PPS to JS(Rare Diseases);
9. PS to DDG(Planning), Dte. GHS.


30/5/2021

(Rajeev Kumar)

Under Secretary to the Government of India

Tele: 011-23063019

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214

NATIONAL POLICY

FOR

RARE DISEASES, 2021

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1. Background

Ministry of Health and family Welfare, Government of India formulated a National Policy for Treatment of Rare Diseases (NPTRD) in July, 2017. Implementation of the policy, however, faced certain challenges. A limiting factor in its implementation was bringing States on board and lack of clarity on how much Government could support in terms of tertiary care. Public Health and Hospitals is primarily a State subject. Stakeholder consultation with the State Governments at the draft stage of formulation of the policy could not be done in an elaborate manner. When the policy was shared with State Governments, issues such as cost effectiveness of interventions for rare disease vis-à-vis other health priorities, the sharing of expenditure between Central and State Governments, flexibility to State Governments to accept the policy or change it according to their situation, were raised by some of the State Governments.

In the circumstances, though framed with best intent, the policy had implementation challenges and gaps, including the issue of cost effectiveness of supporting such health interventions for limited resource situation, which made it not feasible to implement. Given the challenges in implementing the policy, the need for wider consultation and recommendations, a decision was taken to reframe the National Policy for Treatment of Rare Diseases. An Expert Committee was constituted by Ministry of Health and Family Welfare in November, 2018 to review the NPTRD, 2017. The Terms and References of the Expert Committee are given below:

- a. To review the national Policy for Treatment of rare Diseases, 2017 and to suggest amendments/changes as may be required.
- b. To define Rare diseases for India.
- c. To draft National Policy for Rare Diseases.
- d. To suggest vision and strategy in country's context.

Pending reframing the policy, the earlier policy has been kept in abeyance vide a non-statutory Gazette Notification dated 18-12-2018, till the revised policy is issued or till further orders, whichever is earlier.

Based on the report of the Expert Committee and with the approval of the competent authority, the draft National Policy for Rare Diseases, was finalized and placed in the public domain on 13.1.2020 inviting comments/views from all the stakeholders, general public, organisations and States/UTs.

Comments/suggestions received from general public/organisations/stakeholders/States/UTs were referred to DGHS for examination and to submit recommendations. DGHS constituted an Expert Committee to examine the comments/suggestions received. Based on the examination of the comments/suggestions received and recommendations of the same Expert Committee and after further deliberation, the National Policy for Rare Diseases has been finalised.

2. Rare Diseases: Issues & Challenges

The field of rare diseases is complex and heterogeneous. The landscape of rare diseases is constantly changing, as there are new rare diseases and conditions being identified and reported regularly in medical literature. Apart from a few rare diseases, where significant progress has been made, the field is still at a nascent stage. For a long time, doctors, researchers and policy makers were unaware of rare diseases and until very recently there was no real research or public health policy concerning issues related to the field. This poses formidable challenges in development of a comprehensive policy on rare diseases. Nevertheless, it is important to take steps, in the short as well as long term, with the objective of tackling rare diseases in a holistic and comprehensive manner.

2.1 The varying definitions of rare diseases

WHO defines rare disease as often debilitating lifelong disease or disorder with a prevalence of 1 or less, per 1000 population. However, different countries have their own definitions to suit their specific requirements and in context of their own population, health care system and resources. In the US, rare diseases are defined as a disease or condition that affects fewer than 200,000 patients in the country (6.4 in 10,000 people). EU defines rare diseases as a life-threatening or chronically debilitating condition affecting no more than 5 in 10,000 people. Japan identifies rare diseases as diseases with fewer than 50,000 prevalent cases (0.04%) in the country. A summary of the prevalence based definitions of rare diseases used in various countries is tabulated below:

Table 1: Definitions of Rare Disease in different countries

S No.	Country	Prevalence less than per 10,000 population
1	USA	6.4
2	Europe	5.0
3	Canada	5.0
4	Japan	4.0
5	South Korea	4.0
6	Australia	1.0
7	Taiwan	1.0

Source: The I.C. Verma Sub-Committee Report 'Guidelines for Therapy and Management'

The use of varying definitions and diverse terminology can result in confusion and inconsistencies and has implications for access to treatment and for research and

development. According to a study¹ that reviewed and analysed definitions across jurisdictions, most definitions, as discussed above, appear to consider disease prevalence, but other criteria also apply sometimes, such as - disease severity, whether the disease is life-threatening, whether there are alternative treatment options available, and whether it is heritable. The study found that relatively few definitions included qualifiers relating to disease severity and/or a lack of existing treatments, whereas most definitions included a prevalence threshold. The average prevalence thresholds used to define rare diseases ranges among different jurisdictions from 1 to 6 cases/10,000 people, with WHO recommending a prevalence less than 10/10,000 population for defining rare diseases. The study concluded that attempts at harmonising the differing definitions, should focus on standardizing objective criteria such as prevalence thresholds and avoid qualitative descriptors like severity of the disease.

However, it has been contested that disease prevalence alone may also not be an accurate basis for defining rare diseases, as it does not take into account changes in population over time. Hence, some have suggested that a more reliable approach to arriving at a definition could be based on the factors of – a) location - a disease which is uncommon in one country may be quite common in other parts of the world; b) levels of rarity - some diseases may be much more rare than other diseases which are also uncommon; and c) study-ability - whether the prevalence of a disease lends itself to clinical trials and studies.

This underscores the need for further research to better understand the extent of the existing diversity of definitions for rare diseases and to examine the scope of arriving at a definition, which is best suited to the conditions in India. It shall be done on a priority basis as soon as sufficient data is available. Steps have already been taken for creation of a hospital based National Registry for rare diseases in India by ICMR.

1. ¹ Richter, T., Nestler-Parr, S., Babela, R., Khan, Z. M., Tesoro, T., Molsen, E., & Hughes, D. A. (2015). *Rare Disease Terminology and Definitions – A Systematic Global Review: Report of the ISPOR Rare Disease Special Interest Group [Electronic version]. Value in Health, 18, 906-914.* Available at: <https://www.ispor.org/raredisease-terms-definitions.pdf>

2.2 Diagnosis of rare diseases

Early diagnosis of rare diseases is a challenge owing to multiple factors that include lack of awareness among primary care physicians, lack of adequate screening and diagnostic facilities.

Traditional genetic testing includes tests that can only address a few diseases. As a result, physicians most often provide their best guess on which tests are to be done. If the test is negative, further testing will be required using next generation sequencing based tests, or chromosomal microarray which are applicable, but expensive and time-consuming processes with interpretation and counselling issues at times.

There is a lack of awareness about rare diseases in general public as well as in the medical fraternity. Many doctors lack appropriate training and awareness to be able to correctly and timely diagnose and treat these conditions. According to a recent report², it takes patients in United States (US) an average of 7.6 years and patients in United Kingdom (UK) an average of 5.6 years to receive an accurate diagnosis, typically involving as many as eight physicians (four primary care and four specialists). In addition, two to three misdiagnoses are typical before arriving at a final diagnosis. Delay in diagnosis or a wrong diagnosis increases the suffering of the patients exponentially. There is an immediate need to create awareness amongst general public, patients & their families and doctors, training of doctors for early and accurate diagnosis, standardization of diagnostic modalities and development of newer diagnostic and therapeutic tools.

2.3 Challenges in research and development

A fundamental challenge in research and development for the majority of rare diseases is that there is relatively little known about the pathophysiology or the natural history of these diseases. Rare diseases are difficult to research upon as the patient pool

² Rare Disease Impact Report: Insights from patients and the medical community available at: <https://globalgenes.org/wp-content/uploads/2013/04/ShireReport-1.pdf>

is very small and it often results in inadequate clinical experience. Therefore, the clinical explanation of rare diseases may be skewed or partial. The challenge becomes even greater as rare diseases are chronic in nature, where long term follow-up is particularly important. As a result, rare diseases lack published data on long-term treatment outcomes and are often incompletely characterised.

This makes it necessary to explore international and regional collaborations for research, collaborations with the physicians who work on any rare disease and with patient groups and families dealing with the consequences of these disorders. This will help gain a better understanding of the pathophysiology of these diseases, and the therapeutic effects that would have a meaningful impact on the lives of patients. There is also a need to review and where possible modify, clinical trial norms keeping in mind the particular challenges in rare diseases, without compromising on the safety and quality of the drugs or diagnostic tools.

2.4 Challenges in treatment

2.4.1 Unavailability of treatment

Availability and access to medicines are important to reduce morbidity and mortality associated with rare diseases. Despite progress in recent years, effective or safe treatment is not available for most of the rare diseases. Hence, even when a correct diagnosis is made, there may not be an available therapy to treat the rare disease. There are between 7000 - 8000 rare diseases, but less than 5% have therapies available to treat them. About 95% rare diseases have no approved treatment³ and less than 1 in 10 patients receive disease specific treatment. Where drugs are available, they are prohibitively expensive, placing immense strain on resources.

³ [https://www.thelancet.com/journals/landia/article/PIIS2213-8587\(19\)30006-3/fulltext](https://www.thelancet.com/journals/landia/article/PIIS2213-8587(19)30006-3/fulltext)

2.4.2 Prohibitive cost of treatment

As the number of persons suffering from individual rare diseases is small, they do not constitute a significant market for drug manufacturers to develop and bring to market drugs for them. For this reason, rare diseases are also called 'orphan diseases' and drugs to treat them are called "orphan drugs". Where, they do make drugs to treat rare diseases, the prices are extremely high apparently to recoup the cost of research and development. At present very few pharmaceutical companies are manufacturing drugs for rare diseases globally and there are no domestic manufacturers in India except for Food for Special Medical Purposes(FSMP) for small molecule inborn errors of metabolism. Due to the high cost of most therapies, the government has not been able to provide these for free. It is estimated that for a child weighing 10 kg, the annual cost of treatment for some rare diseases, may vary from Rupees 10 Lakhs to more than 1 crore per year with treatment being lifelong and drug dose and cost increasing with age and weight.

Countries have dealt with this unique problem of high cost through various means that were suited to their local needs. Instruments like the Orphan Drug Act (ODA) in US & Canada, provide incentives to drug manufacturers to encourage them to manufacture drugs for rare diseases. The economic incentives & safeguards offered under the Act ensure benefits to the local patients. However, the exorbitant prices of drugs for rare diseases has led to concerns even in the developed countries about maintaining sustainability of the rare diseases funding/reimbursement programmes. The exorbitant prices have led to calls for transparency in setting prices of drugs and for price control and have even prompted scrutiny and congressional inquiries.

3. The Indian Scenario

Data on how many people suffer from different diseases that are considered rare globally, is lacking in India. The cases identified so far have been diagnosed at tertiary hospitals. The lack of epidemiological data on incidence and prevalence of rare diseases impedes understanding of the extent of the burden of rare diseases and development of a

definition. It also hampers efforts to arrive at correct estimation of the number of persons suffering from these diseases and describe their associated morbidity and mortality. In such a scenario, the economic burden of most rare diseases is unknown and cannot be adequately estimated from the existing data sets.

Although extremely challenging, considering the complexity of various diseases and the difficulty in diagnosis, there is a clear need to undertake systematic epidemiological studies to ascertain the number of people suffering from rare diseases in India.

So far only limited number of diseases has been recorded in India from tertiary care hospitals that are globally considered as rare diseases though ambit may encompass from 7000 to 8000 disorders. The commonly reported diseases include Primary immunodeficiency disorders, Lysosomal storage disorders (Gaucher's disease, Mucopolysaccharidoses, Pompe disease, fabry disease etc.) small molecule inborn errors of metabolism (Maple Syrup urine disease, organic acidemias, etc.), Cystic Fibrosis, osteogenesis imperfecta, certain forms of muscular dystrophies and spinal muscular atrophy, etc.

4. Experiences from other countries:

While preparing the policy for rare diseases in India, policies of other countries have been reviewed. In United States of America, development of drugs for rare disease is sought to be encouraged through the Orphan Drugs Act, which incentivises industry by way of market exclusivity, grants to researchers and tax incentives on expenditure incurred during evaluation of drugs for their therapeutic potential. However, critics have pointed out that pharmaceutical companies have taken advantage of this arrangement and 'gamed the system' to maximise profits. The European Joint Programme on Rare Disease mostly focuses on research. National Health Service (NHS) England, for example, provides that the treatment for Spinal Muscular Atrophy (SMA) will be made available to the youngest and most severely-affected (SMA Type 1) patients immediately by Biogen (The pharmaceutical company that manufactures treatment for SMA), with NHS England offering funding on

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National Institute for Health and Care Excellence (NICE) publication of final guidance. In Singapore, a fund - Rare Disease Fund – has been created to fund five medicines to treat three rare disease conditions. In Malaysia and Australia subsidised access for eligible patients is provided for expensive and lifesaving drugs.

5. Need to balance competing priorities of public health in resource constrained settings

Rare diseases place a major economic burden on any Country and especially in resource-constrained settings. The financial capacity to support exorbitant cost of treatment, is an important consideration in public health policy development with reference to treatment for rare diseases. In resource-constrained settings, it is pertinent to balance competing interests of public health for achieving optimal outcome for the resources allocated. As resources are limited and have multiple uses, the policy makers have to make choice of prioritizing certain set of interventions over others- the appropriate choice is then to support those interventions that would provide more number of healthy life years for given sum of money while simultaneously looking at the equity i.e., interventions that benefit poor who cannot afford healthcare are prioritized. Thus, interventions that address health problems of a much larger number of persons by allocating a relatively smaller amount are prioritized over others such as funding treatment of rare diseases where much greater resources will be required for addressing health problems of a far smaller number of persons.

Hence, any policy on rare diseases needs to be considered in the context of the available scarce resources and the need for their utmost judicious utilization for maximizing the overall health outcomes for the whole of society measured in terms of increase of healthy life years.

6. Definition of Rare Diseases:

6.1 There is no universal or standard definition of rare disease. A disease that occurs infrequently is generally considered a rare disease, and it has been defined by different countries in terms of prevalence – either in absolute terms or in terms of prevalence per 10,000 population. A country defines a rare disease most appropriate in the context of its own population, health care system and resources.

6.2 As mentioned above, India faces the limitation of lack of epidemiological data to be able to define rare diseases in terms of prevalence or prevalence rate, which has been used by other countries. To overcome this, a hospital based National Registry for Rare Diseases has been initiated by ICMR by involving centers across the Country that are involved in diagnosis and management of Rare Diseases. This will yield much needed epidemiological data for rare diseases. In the absence of epidemiological data on diseases considered as rare in other countries, it is not possible to prescribe threshold prevalence rates to define a disease condition as rare.

Till the time such data is available and the Country arrives at a definition of a rare disease based on prevalence data, the term rare diseases, for the purpose of this policy, shall construe the following groups of disorders identified and categorized by experts based on their clinical experience:

Group 1: Disorders amenable to one-time curative treatment:

a) Disorders amenable to treatment with Hematopoietic Stem Cell Transplantation (HSCT) –

- i. Such Lysosomal Storage Disorders (LSDs) for which Enzyme Replacement Therapy (ERT) is presently not available and severe form of Mucopolysaccharoidosis (MPS) type I within first 2 years of age.*
- ii. Adrenoleukodystrophy (early stages), before the onset of hard neurological signs.*

- iii. Immune deficiency disorders like Severe Combined Immunodeficiency (SCID), Chronic Granulomatous disease, Wiskot Aldrich Syndrome, etc.
- iv. Osteopetrosis
- v. Fanconi Anemia

b) Disorders amenable to organ transplantation

i. Liver Transplantation -Metabolic Liver diseases:

- a. Tyrosinemia,
- b. Glycogen storage disorders (GSD) I, III and IV due to poor metabolic control, multiple liver adenomas, or high risk for Hepatocellular carcinoma or evidence of substantial cirrhosis or liver dysfunction or progressive liver failure,
- c. MSUD (Maple Syrup Urine Disease),
- d. Urea cycle disorders,
- e. Organic acidemias.

ii. Renal Transplantation-

- a. Fabry disease
- b. Autosomal recessive Polycystic Kidney Disease (ARPKD),
- c. Autosomal dominant Polycystic Kidney Disease (ADPKD) etc.

- iii. Patients requiring combined liver and kidney transplants can also be considered if the same ceiling of funds is maintained. (Rarely Methyl Malonic aciduria may require combined liver & Kidney transplant) etc.

Group 2: Diseases requiring long term / lifelong treatment having relatively lower cost of treatment and benefit has been documented in literature and annual or more frequent surveillance is required:

a) Disorders managed with special dietary formulae or Food for special medical purposes (FSMP)

- i) Phenylketonuria (PKU)
- ii) Non-PKU hyperphenylalaninemia conditions
- iii) Maple Syrup Urine Disease (MSUD)
- iv) Tyrosinemia type 1 and 2
- v) Homocystinuria
- vi) Urea Cycle Enzyme defects
- vii) Glutaric Aciduria type 1 and 2
- viii) Methyl Malonic Acidemia
- ix) Propionic Acidemia
- x) Isovaleric Acidemia
- xi) Leucine sensitive hypoglycemia
- xii) Galactosemia
- xiii) Glucose galactose malabsorption
- xiv) Severe Food protein allergy

b) Disorders that are amenable to other forms of therapy (hormone/ specific drugs)

- i) NTBC for Tyrosinemia Type 1
- ii) Osteogenesis Imperfecta – Bisphosphonates therapy
- iii) Growth Hormone therapy for proven GH deficiency, Prader Willi Syndrome and Turner syndrome, Noonan syndrome.
- iv) Cystic Fibrosis- Pancreatic enzyme supplement
- v) Primary Immune deficiency disorders -Intravenous immunoglobulin and sub cutaneous therapy (IVIG) replacement eg. X-linked agammaglobulinemia etc.

vi) Sodium Benzoate, arginine, citrulline, phenylacetate (Urea Cycle disorders), carbaglu, Megavitamin therapy (Organic acidemias, mitochondrial disorders)

vii) Others - Hemin (Panhematin) for Acute Intermittent Porphyria, High dose Hydroxocobalamin injections (30mg/ml formulation – not available in India and hence expensive if imported)

viii) Large neutral aminoacids, mitochondrial cocktail therapy, Sapropterin and other such molecules of proven clinical management in a subset of disorders

Group 3: Diseases for which definitive treatment is available but challenges are to make optimal patient selection for benefit, very high cost and lifelong therapy.

3a) Based on the literature sufficient evidence for good long-term outcomes exists for the following disorders

1. Gaucher Disease (Type I & III {without significant neurological impairment})
2. Hurler Syndrome [Mucopolysaccharisosis (MPS) Type I] (attenuated forms)
3. Hunter syndrome (MPS II) (attenuated form)
4. Pompe Disease (Both infantile & late onset diagnosed early before development of complications)
5. Fabry Disease diagnosed before significant end organ damage.
6. MPS IVA before development of disease complications.
7. MPS VI before development of disease complications.
8. DNAase for Cystic Fibrosis.

3b) For the following disorders for which the cost of treatment is very high and either long term follow up literature is awaited or has been done on small number of patients

1. Cystic Fibrosis (Potentiators)

2. *Duchenne Muscular Dystrophy (Antesence oligoneucleotides, PTC)*
3. *Spinal Muscular Atrophy (Antisense oligonucleotides both intravenous & oral & gene therapy)*
4. *Wolman Disease*
5. *Hypophosphatasia*
6. *Neuronal ceroid lipofuscinosis*

6.3. The list of diseases under Group 1, Group 2 and Group 3 are not exhaustive and will be reviewed periodically based on updated scientific data by the Technical Committee.

7. Policy Direction

The policy aims at lowering the incidence and prevalence of rare diseases based on an integrated and comprehensive preventive strategy encompassing awareness generation, premarital, post-marital, pre-conception and post-conception screening and counselling programmes to prevent births of children with rare diseases, and, within the constraints on resources and competing health care priorities, enable access to affordable health care to patients of rare diseases which are amenable to one-time treatment or relatively low cost therapy.

Considering the limited data available on rare diseases, and in the light of competing health priorities, the focus would be on prevention of rare diseases as a priority for all the three groups of rare diseases identified by Experts. Public Health and hospitals being a State subject, the Central Government would encourage & support the States in their endeavour towards screening and prevention of rare diseases through Centres of Excellence under Rare Disease Policy and Nidan Kendras under Department of Biotechnology.

8. Prevention & Control of Rare Diseases:

8.1 Capacity building of health professionals

The Central Government will work with the State governments to build capacity of health professionals at various levels. The content of such capacity building would be based on the roles of various health professionals. The Centres of Excellence would develop Standard Operating Protocols to be used at various levels of care for patients with rare diseases to improve early diagnosis, better care coordination and quality of life.

8.2 Prevention at different levels

Though in the last two decades, due to advancement in technologies, understanding of the pathophysiological mechanisms of rare genetic disorders has somewhat improved, yet the treatment modalities are few and the available therapies may not lead to 'cure'. More importantly these are exorbitantly costly and not universally available & accessible. Accordingly, prevention needs to be the focus for all genetic disorders. The prevention of genetic disorders can be done at multiple levels. For application of these strategies, the first step is to build the capacity of health professionals and increase awareness in the population at large about the prevalence of such diseases and prevention measures. Frontline workers will be adequately capacitated for screening of rare diseases. Adequate IEC material will be designed and made available across multiple levels of the health care pyramid as this forms a basic pillar of tackling the issue of limited awareness.

8.2.1: Primary Prevention: This aims at preventing the occurrence of the disease, i.e., preventing birth of an affected child. Though not always feasible, this strategy yields the highest returns in terms of decreasing the incidence & prevalence of rare disorders in the population in the long run. Some of the strategies can be as follows:

Examples include avoidance of pregnancy in advanced age, or any other rare monogenic disorder by not marrying a carrier, carrier couples not reproducing etc., but these are not feasible options in the real world scenario. So in most situations the feasible preventive

strategy is secondary prevention. However, a simple checklist will be made available to primary health care providers in the health and wellness clinics to identify a couple at risk based on disease in a previous sib or family history of that disorder.

8.2.2: Secondary prevention: This strategy focuses on avoiding the birth of affected fetus (prenatal screening and prenatal diagnosis), early detection of the disorders, appropriate medical intervention to ameliorate or minimize the manifestations (newborn screening).

a) Prenatal screening: The common screening methods presently recommended for all pregnancies include biochemical screening and ultrasonography for chromosomal disorders like Down syndrome etc. and ultrasonography for other structural defects. In the context of rare diseases objective of the prenatal screening and diagnosis is to identify the high risk mothers for having an affected fetus with a rare disease. These mothers can be identified based on the family history (previous affected child or affected relative with a known or suspected genetic disorder). Based on the suspected disease a targeted screening of the affected child or couple for a specific disorder or a carrier testing for monogenic disorders using next generation sequencing technique can be offered, the later being presently expensive.

b) Prenatal diagnosis by invasive testing (e.g., by Chorionic villus sampling and amniocentesis): is possible for any single-gene disorder if the disease causing variant in the gene / enzyme defect is known and for any chromosomal abnormality. Most common indications are known single gene disorders or chromosomal abnormality in a previous affected child in the family. These tests can also be offered if the married couple is found to be carrier for any single gene disorder and mutations have been identified in the couple. Now a day, prenatal diagnosis for above mentioned disorders are widely available in India at many institutions. The invasive procedures are performed by obstetricians and fetal

medicine experts. These procedures however carry a small risk of fetal loss which is very low if done by experienced specialists. This has to be explained to the family before the procedure. Cost would primarily depend on the type of the test to be performed on the sample. If the fetus is found to be affected, the couple has the option for termination of pregnancy, the legal age of which in India has been increased to 24 weeks of pregnancy.

c) **Newborn screening (NBS):** is the best example of secondary prevention in which the babies are screened with in few days of birth before symptoms of the disease manifest and treatment is initiated which prevents morbidity and mortality. In the developed world NBS is being offered for many rare disorders particularly the treatable ones (e.g., LSDs, SCID) apart from the common disorders.

d) **Early postnatal diagnosis and treatment:** before development of severe manifestations /complications which are irreversible is also included in secondary prevention for disorders amenable to therapy which would require increasing awareness and better availability of diagnostics. Timely referral of the suspected patients & their families to appropriate facilities that are equipped to make a correct diagnosis and where indicated, initiate treatment is the key. Genetic testing will also be augmented by laboratories under the National Genomics Core funded by the Department of Biotechnology and Institute of Genomics and Integrative Biology (IGIB) & Centre for Cellular and Molecular Biology (CCMB) under CSIR.

8.2.3: Tertiary prevention refers to provision of better care and medical rehabilitation to those rare disease patients who present at an advanced stage of the disease. It encompasses providing best supportive care to the affected patients with various rare disorders including the ones for which no specific treatment is available. This would improve quality of life of affected individuals and families. Supportive care includes developmental assessment and intervention including early stimulation and behavioural

intervention, physical therapy and rehabilitation, provision of visual and hearing aids and above all emotional and psychological support to affected individuals and families.

8.2.4: Optimal screening and diagnosis strategy: Considering the competing priorities within available resources, universal screening of all pregnancies and/or all newborns in the country for all rare disorders is not feasible. The policy recommends a screening and diagnostic strategy wherein those pregnant women in whom there is a history of a child born with a rare disease and that rare disease diagnosis has been confirmed, would be offered prenatal screening test(s) through amniocentesis and / or chorionic villi sampling. This strategy is in sync with the policy direction of reducing the incidence of rare diseases in the population. In cases where, the diagnosis could not be established during the prenatal period, it would be imperative to offer to the newborn or the infant as the case may be and would include newborn screening for (a) small molecule Inborn Errors of Metabolism by liquid chromatography – tandem mass spectrometry (LC-MS/MS), (b) diagnosis of SCID by T cell receptor excision circles (TREC) and (c) diagnosis of lysosomal storage disorders (LSDs) by microfluids / LC-MS/ MS. (d) diagnosis of disorders by newer but economical molecular diagnostic platforms.

9. Centres of Excellence (COE) and Nidan Kendras

9.1 The Government will notify selected Centres of Excellence, which will be premier Government tertiary hospitals with facilities for diagnosis, prevention and treatment of rare diseases. To begin with the following institutes would be notified as Centers of Excellence for Rare Diseases:

- a) All India Institute of Medical Sciences, New Delhi
- b) Maulana Azad Medical College, New Delhi
- c) Sanjay Gandhi Post Graduate Institute of Medical Sciences, Lucknow
- d) Post Graduate Institute of Medical Education and Research, Chandigarh
- e) Centre for DNA Fingerprinting & Diagnostics, Hyderabad
- f) King Edward Medical Hospital, Mumbai

- g) Institute of Post-Graduate Medical Education and Research, Kolkata
- h) Center for Human Genetics (CHG) with Indira Gandhi Hospital, Bengaluru

However, more Centres of Excellence can be added for regional outreach if they are found to be suitable in terms of infrastructure and human resources based on recommendations of technical committee.

9.2 The responsibilities and activities of the COEs would be as follows:

- Education & Training at all levels
- Screening – Antenatal, neonatal (specified disorders), High risk screening (both antenatal & in newborns and children)
- Diagnostics- Cytogenetic, molecular, Metabolic
- Prevention by prenatal screening & diagnosis
- Research in the area of low cost diagnostics & therapeutics.
- Treatment of rare diseases.

9.3 The proposed COEs shall be given one-time financial support upto a ceiling of Rs 5 crore for procurement of equipment as per individual centers need for strengthening patient care services for screening, diagnosis and prevention (prenatal diagnosis) of rare diseases based on a gap analysis. The list of equipments which are likely to be useful for these activities is annexed.

9.4 These Centre of Excellence will take the required decision for treatment and fund allocation on rare diseases cases within 02 weeks of receiving the fresh application.

9.5 Nidan Kendras: Nidan Kendras have been set up by Department of Biotechnology (DBT) under Unique Methods of Management and treatment of Inherited Disorders (UMMID) project for genetic testing and counseling services. These Nidan Kendras will be performing screening, genetic testing and counseling for rare diseases. Nidan Kendras possessing the facility for treatment may do so under the guidance and supervision of a CoE.

List of Nidan Kendras is given below:

- Lady Hardinge Medical College (LHMC), Delhi
- Nizam's Institute of Medical Sciences (NIMS), Hyderabad, Telangana
- All India Institute of Medical Sciences (AIIMS), Jodhpur
- Army Hospital Research & Referral, Delhi
- Nil Ratan Sircar (NRS) Medical College and Hospital, Kolkata

Currently Nidan Kendras / Mentor Institutes are supporting aspirational districts for screening of rare diseases. List of aspirational districts covered under the programme is given below:

Name of the Mentor Institute	Aspirational District	State
LHMC, New Delhi	Mewat	Haryana
CDFD, Hyderabad	Yadgir	Karnataka
AIIMS, New Delhi	Haridwar	Uttarakhand
CMC, Vellore	Washim	Maharashtra
MAMC, New Delhi	Ranchi / Bokaro	Jharkhand
SGPGIMS, Lucknow	Shrawasti	Uttar Pradesh
NIIH (KEM hospital campus), Mumbai	Nandurbar	Maharashtra

More aspirational districts will be covered in future either by setting up of more Nidan Kendras or by adopting more than one aspirational districts by existing Nidan Kendras.

10. Government of India support in treatment

The following initiatives shall be taken for patients of Rare Diseases:

- Financial support upto Rs. 20 lakh under the Umbrella Scheme of Rashtriya Arogya Nidhi shall be provided by the Central Government for treatment, of those rare diseases that require a one-time treatment (diseases listed under Group 1). Beneficiaries for such financial assistance would not be limited to BPL families, but extended to about 40% of the population, who are eligible as per

norms of Pradhan Mantri Jan Arogya Yojana, for their treatment in Government tertiary hospitals only.

- ii. State Governments can consider supporting patients of such rare diseases that can be managed with special diets or hormonal supplements or other relatively low cost interventions (Diseases listed under Group 2).
- iii. Keeping in view the resource constraints, and a compelling need to prioritize the available resources to get maximum health gains for the community/population, the Government will endeavour to create alternate funding mechanism through setting up a digital platform for voluntary individual and corporate donors to contribute to the treatment cost of patients of rare diseases.
- iv. **Voluntary crowd-funding for treatment**

Keeping in view the resource constraint and competing health priorities, it will be difficult for the Government to fully finance treatment of high cost rare diseases. The gap can however be filled by creating a digital platform for bringing together notified hospitals where such patients are receiving treatment or come for treatment, on the one hand, and prospective individual or corporate donors willing to support treatment of such patients. The notified hospitals will share information relating to the patients, diseases from which they are suffering, estimated cost of treatment and details of bank accounts for donation/contribution through online system. Donors will be able to view the details of patients and donate funds to a particular hospital. This will enable donors from various sections of the society to donate funds, which will be utilized for treatment of patients suffering from rare diseases, especially those under Group 3. Conferences will be organised with corporate sector companies to motivate them to donate generously through digital platform. Ministry of Corporate Affairs will be requested to encourage PSUs and corporate houses to contribute as per the Companies Act as well as the provisions of the Companies (Corporate Social Responsibility Policy) Rules, 2014 (CSR Rules). Promoting health

care including preventive health care is included in the list in the Schedule for CSR activities.

Treatment cost of the patient will be first charge on this fund. Any leftover fund after meeting treatment cost can be utilized for research purpose also.

11. Development of manpower

Following initiatives will be taken for strengthening of manpower:

- State Governments will be requested to create Department of Medical Genetics at least in one medical colleges in the State for imparting education and increasing awareness amongst health care professionals.
- Services of Nidan Kendras set up under Department of Biotechnology will also be utilised for training of medical practitioners and staff for screening for rare diseases.

12. Constitution of Consortium

(a) **Consortium of Centres of Excellence** so created will synchronize prevention and treatment efforts. AIIMS, Delhi will be the nodal hospital to coordinate with other Centres of Excellence for various activities relating to prevention and treatment of rare disease.

(b) **National Consortium for Research and Development on therapeutics for Rare Diseases:** National Consortium can be provided with an expanded mandate to include research & development, technology transfer and indigenization of therapeutics for rare diseases. It will be convened by Department of Health Research (DHR) with ICMR as a member.

13. Increasing affordability of drug related to rare diseases

(a) Research & Development activities on rare diseases

Indian Council of Medical Research (ICMR), Department of Biotechnology, Department of Pharmaceuticals, Department of science and Technology and Council for Scientific Industrial & Research will be requested to promote research and development in the field of rare diseases for diagnosis and treatment of rare diseases.

Creation of an integrated research pipeline to start the development of new drugs, for which pharmaceutical companies would be encouraged and research organizations as well as funding agencies would be involved in this important endeavour. Research for repurposing the drugs and use of biosimilar would be encouraged. Approval for new drugs and decision related to trials will continue to be provided by Drugs Controller General of India under the New Drugs and Clinical Trial Rules, 2019.

(b) Ministry of Finance will be requested for reduction in custom duties on import of medicines related to rare diseases.

(c) Ministry of Chemicals and Fertilizers, Department of Pharmaceutical (DoP), National Pharmaceutical Pricing Authority (NPPA) shall take measures to document and make publicly available the prices of drugs for rare diseases and work towards affordability of drugs for rare diseases, in consultation with the Ministry of Health and Family Welfare.

(d) Measures for creating conducive environment for indigenous manufacturing of drugs for rare diseases would be taken. Department of Pharmaceuticals, , Department for Promotion of Industry and Internal Trade (DPIIT) will be requested to promote local development and manufacture of drugs for rare diseases at affordable prices and take legal/legislative measures for creating conducive environment for indigenous manufacturing of drugs for rare diseases at affordable prices. PSUs would be encouraged for local manufacturing of drugs for rare diseases.

14. Implementation strategy

Keeping in view lack of availability of epidemiological data on rare diseases, constraints on resources and competing health priorities, the focus of the Government will be on the following:

- i. The Government will have a hospital based National Registry for Rare Diseases at ICMR with the objective of creating a database of various rare diseases. Steps have already been taken in this direction by ICMR. Over a period of time, the registry is expected to yield information on hospital based data and disease burden.
- ii. The Government shall take steps to create awareness amongst all the levels of health care personnel as well as general public towards the rare diseases. This will encourage people to seek pre-marital genetic counselling, identification of high-risk couples & families and also result in prevention of births as well as early detection of cases of rare diseases. Simple standard protocols/algorithms would be developed for screening and diagnosis in order to avoid missing cases and provide best possible management
- iii. Public Health and hospitals being a State subject, the Central Government shall encourage and support the State Governments in implementation of a targeted preventive strategy.
- iv. The Government shall provide financial assistance upto Rs. 20.00 lakh (under the Umbrella Scheme of Rashtriya Arogya Nidhi) to the entitled population, as per PMJAY norms, for their treatment in Government tertiary hospital, for rare diseases amenable to one-time treatment (identified under Group 1).
- v. The State Governments may undertake treatment of disorders managed with special dietary formulae or food for special medical purposes (FSMP) and Disorders that are amenable to other forms of therapy (hormone/ specific drugs)- diseases covered under Group 2.
- vi. The Government shall notify selected Centres of Excellence at premier government hospitals for comprehensive management of rare diseases. The Centres of Excellence will be provided one time grant subject to maximum of Rs. 5 crore

each for infrastructure development for screening, tests, treatment, if such infrastructure is not available.

- vii. The Government shall create a digital platform for bringing together notified Centres of Excellence where patients of rare diseases can receive treatment or come for treatment, on the one hand and prospective voluntary individual or corporate donors willing to support treatment of such patients. Funds received through this mechanism will be utilized for treatment of patients suffering from rare diseases.
- viii. In order to maintain transparency of transactions in provision of funding under RAN/ crowd funding etc., the Centres of Excellence receiving the funds should have linkages with the ICMR registry.
- ix. The Government shall facilitate the creation of an enabling environment that promotes research & development of diagnostic and therapeutic modalities within the Country. Consortium of Centres of Excellence shall be created so that research efforts are synchronized. AIIMS, Delhi will be nodal hospital to coordinate with other Centres of Excellence for various activities.
- x. State Governments will be requested to create Department of Medical Genetics at least in one medical college in the State for imparting education and increasing awareness amongst health care professionals. This will strengthen manpower base in the country for managing Rare Diseases.
- xi. Department of Pharmaceuticals, Department for Promotion of Industry and Internal Trade (DPIIT) will be requested to promote local development and manufacture of drugs for rare diseases by public and private sector pharmaceutical companies at affordable prices and take legal/legislative measures for creating conducive environment for indigenous manufacturing of drugs for rare diseases at affordable prices. PSUs could also be encouraged for local manufacturing of drugs for rare diseases.
- xii. Ministry of Finance will be requested for reduction in custom duties on import of medicines related to rare diseases.

AnnexureSuggestive List of Equipment, which may be required for strengthening of patient services at Centres of Excellence for screening, diagnosis and ~~prevention~~ (prenatal diagnosis) of rare disease.

- Cytogenetic workstation with software with Fluorescent in situ hybridization
- Multimode readers for both ELISA and fluorescent enzyme assays
- DNA Sequencer with 8 capillary sequencer
- Mi Seq next generation sequencer
- Next Seq next generation sequencer
- Liquid chromatography Mass Spectroscopy (Tandem Mass Spectrometry)
- HPLC (quaternary pump high Performance Liquid Chromatography)
- GCMS (gas chromatography Mass Spectrometry)
- Microfluidics platform
- Real Time PCR (96 well format) for real time polymerase chain reaction
- High throughput rNA and DNA extraction systems
- Quality Check stations and microtips station
- Chromosomal Micro array platform
- Newborn Screening platform for fluoroimmunoassay
- Antenatal screening equipment (one stop screening for pre-eclampsia and chromosomal aneuploidies)
- Bio-informatics set up for Next generation data analysis using High End desktop
- Eonis tm system for DNA based newborn screening for rare disorders
- Capillary Electrophoresis system for newborn screening of hemoglobinopathies
- Upgradation of existing equipment's may also be considered to save costs benefiting a larger section
- Any other with permission of the MOHFW with proper justification and as decided by a technical committee of experts set up by MoHFW.